

## PATENT COOPERATION TREATY

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REC'D. 04 NOV 2004

INTERNATIONAL PRELIMINARY EXAMINATION REPORT

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(PCT Article 36 and Rule 70)

Applicant's or agent's file reference 43000.014810	<b>FOR FURTHER ACTION</b> See Notification of Transmittal of International Preliminary Examination Report (Form PCT/IPEA/416)	
International application No. PCT/US03/12225	International filing date (day/month/year) 17 April 2003 (17.04.2003)	Priority date (day/month/year) 17 April 2002 (17.04.2002)
International Patent Classification (IPC) or national classification and IPC IPC(7): C12N 15/864, 7/00, 7/01, 5/10; A61K 48/00 and US Cl.: 435/320.1, 325, 366, 69.1, 455, 456, 457; 424/93.1, 93.2, 93.6		
Applicant UNIVERSITY OF FLORIDA		
<p>1. This international preliminary examination report has been prepared by this International Preliminary Examining Authority and is transmitted to the applicant according to Article 36.</p> <p>2. This REPORT consists of a total of <u>4</u> sheets, including this cover sheet.</p> <p><input type="checkbox"/> This report is also accompanied by ANNEXES, i.e., sheets of the description, claims and/or drawings which have been amended and are the basis for this report and/or sheets containing rectifications made before this Authority (see Rule 70.16 and Section 607 of the Administrative Instructions under the PCT).</p> <p>These annexes consist of a total of ___ sheets.</p> <p style="text-align: right;">EPO - DG 1</p>		
<p>3. This report contains indications relating to the following items:</p> <p style="text-align: right;">U 3. 12. 2004</p> <p style="text-align: center;">(107)</p> <p>I <input checked="" type="checkbox"/> Basis of the report</p> <p>II <input type="checkbox"/> Priority</p> <p>III <input checked="" type="checkbox"/> Non-establishment of report with regard to novelty, inventive step and industrial applicability</p> <p>IV <input type="checkbox"/> Lack of unity of invention</p> <p>V <input checked="" type="checkbox"/> Reasoned statement under Article 35(2) with regard to novelty, inventive step or industrial applicability; citations and explanations supporting such statement</p> <p>VI <input type="checkbox"/> Certain documents cited</p> <p>VII <input type="checkbox"/> Certain defects in the international application</p> <p>VIII <input type="checkbox"/> Certain observations on the international application</p>		
Date of submission of the demand 13 November 2003 (13.11.2003)	Date of completion of this report 14 August 2004 (14.08.2004)	
Name and mailing address of the IPEA/US Mail Stop PCT, Atto: IPEA/US Commissioner for Patents P.O. Box 1450 Alexandria, Virginia 22313-1450 Facsimile No. (703) 305-3230	Authorized officer David Guzo <i>Janice Ford</i> Telephone No. (703) 308-0196	

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# INTERNATIONAL PRELIMINARY EXAMINATION REPORT

International application No.  
PCT/US03/12225

## I. Basis of the report

### 1. With regard to the elements of the international application:\*

- ☒ the international application as originally filed.
- ☒ the description:  
pages 1-189 as originally filed  
pages NONE filed with the demand  
pages NONE filed with the letter of \_\_\_\_\_
- ☒ the claims:  
pages 190-199 as originally filed  
pages NONE as amended (together with any statement) under Article 19  
pages NONE filed with the demand  
pages NONE filed with the letter of \_\_\_\_\_
- ☒ the drawings:  
pages 1-10 as originally filed  
pages NONE filed with the demand  
pages NONE filed with the letter of \_\_\_\_\_
- ☐ the sequence listing part of the description:  
pages NONE as originally filed  
pages NONE filed with the demand  
pages NONE filed with the letter of \_\_\_\_\_

### 2. With regard to the language, all the elements marked above were available or furnished to this Authority in the language in which the international application was filed, unless otherwise indicated under this item.

These elements were available or furnished to this Authority in the following language \_\_\_\_\_ which is:

- ☐ the language of a translation furnished for the purposes of international search (under Rule 23.1(b)).
- ☐ the language of publication of the international application (under Rule 48.3(b)).
- ☐ the language of the translation furnished for the purposes of international preliminary examination (under Rules 55.2 and/or 55.3).

### 3. With regard to any nucleotide and/or amino acid sequence disclosed in the international application, the international preliminary examination was carried out on the basis of the sequence listing:

- ☐ contained in the international application in printed form.
- ☐ filed together with the international application in computer readable form.
- ☒ furnished subsequently to this Authority in written form.
- ☒ furnished subsequently to this Authority in computer readable form.
- ☐ The statement that the subsequently furnished written sequence listing does not go beyond the disclosure in the international application as filed has been furnished.
- ☐ The statement that the information recorded in computer readable form is identical to the written sequence listing has been furnished.

### 4. ☐ The amendments have resulted in the cancellation of:

- ☐ the description, pages NONE
- ☐ the claims, Nos. NONE
- ☐ the drawings, sheets/fig. NONE

### 5. ☐ This report has been established as if (some of) the amendments had not been made, since they have been considered to go beyond the disclosure as filed, as indicated in the Supplemental Box (Rule 70.2(c)).\*\*

\* Replacement sheets which have been furnished to the receiving Office in response to an invitation under Article 14 are referred to in this report as "originally filed" and are not annexed to this report since they do not contain amendments (Rules 70.16 and 70.17).

\*\* Any replacement sheet containing such amendments must be referred to under item 1 and annexed to this report.

# INTERNATIONAL PRELIMINARY EXAMINATION REPORT

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## III. Non-establishment of opinion with regard to novelty, inventive step and industrial applicability

1. The question whether the claimed invention appears to be novel, to involve an inventive step (to be non-obvious), or to be industrially applicable have not been and will not be examined in respect of:

- ☐ the entire international application,  
☒ claims Nos. 35-43

because:

- ☐ the said international application, or the said claim Nos. \_\_\_\_\_ relate to the following subject matter which does not require international preliminary examination (*specify*):

- ☒ the description, claims or drawings (*indicate particular elements below*) or said claims Nos. 35-43 are so unclear that no meaningful opinion could be formed (*specify*):

Claims 35-43 will not be examined because they are dependent claims and are not drafted in accordance with the second and third sentences of Rule 6.4(a).

- ☐ the claims, or said claims Nos. \_\_\_\_\_ are so inadequately supported by the description that no meaningful opinion could be formed.  
☐ no international search report has been established for said claims Nos. \_\_\_\_\_

2. A meaningful international preliminary examination cannot be carried out due to the failure of the nucleotide and/or amino acid sequence listing to comply with the standard provided for in Annex C of the Administrative Instructions:

- ☐ the written form has not been furnished or does not comply with the standard.  
☐ the computer readable form has not been furnished or does not comply with the standard.

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## V. Reasoned statement under Rule 66.2(a)(ii) with regard to novelty, inventive step or industrial applicability; citations and explanations supporting such statement

### 1. STATEMENT

Novelty (N)	Claims <u>1-34 and 44-51</u>	YES
	Claims <u>NONE</u>	NO
Inventive Step (IS)	Claims <u>1-34 and 44-51</u>	YES
	Claims <u>NONE</u>	NO
Industrial Applicability (IA)	Claims <u>1-34 and 44-51</u>	YES
	Claims <u>NONE</u>	NO

### 2. CITATIONS AND EXPLANATIONS

Claims 1-34 and 44-51 meet the criteria set out in PCT Article 33(2)-(3), because the prior art does not teach or fairly suggest the claimed adeno-associated viral (AAV) vectors comprising a first polynucleotide comprising a first nucleic acid segment that encodes an AAV capsid protein that comprises an exogenous amino acid sequence that binds to a mammalian lipoprotein receptor as well as methods of use of said AAV vectors to target specific mammalian cells or cell types or use of said AAV vectors to treat symptoms of disease in mammalian subjects by delivering a therapeutic gene to target cells *in vivo*.

It is noted that claims 4-7 and 11-13 were previously not searched because said claims recite specific SEQ ID NO:s and a suitable sequence listing was not filed. However, because a computer readable form and paper copy of the sequence listing was filed with the demand, said claims were searched and Smith et al. (US 6,344,436) is newly cited as teaching generation and use of peptide-macromolecule complexes to deliver genes of interest to target cells. Smith et al. does not teach use of AAV vectors having chimeric capsid proteins with an exogenous sequence and does not teach or suggest the claimed invention.

Claims 1-34 and 44-51 meet the criteria set out in PCT Article 33(4), and thus possess industrial applicability because the subject matter claimed can be made or used in industry.

#### NEW CITATIONS

US 6,344,436 B1 (SMITH et al.) 05 February 2002, see columns 2-5.